(19) World Intellectual Property Organization International Bureau





(10) International Publication Number

WO 2005/053601 A3

PCT

(43) International Publication Date 16 June 2005 (16.06.2005)

(51) International Patent Classification:

C12N 5/10 (2006.01) *C12N 5/00* (2006.01) *C12N 5/02* (2006.01) *C12N 5/08* (2006.01) *C12N 5/06* (2006.01) *C12N 15/63* (2006.01)

(21) International Application Number:

PCT/IL2004/001046

(22) International Filing Date:

15 November 2004 (15.11.2004)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

60/525,883

US 1 December 2003 (01.12.2003)

(71) Applicant (for all designated States except US): TECH-NION RESEARCH & DEVELOPMENT FOUNDA-TION LTD. [IL/IL]; Senate House, Technion City, 32000 Haifa (IL).

- (72) Inventors; and
- (75) Inventors/Applicants (for US only): AMIT, Michal [IL/IL]; 261 Yuvalim, 20142 Misgav (IL). IT-SKOVITZ-ELDOR, Joseph [IL/IL]; 42 SheErit HaPleta Street, 34987 Haifa (IL).
- gin Street, 52 521 Ramat Gan (IL).

(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM,

TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM,

(84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IS, IT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

ZW.

- with international search report
- before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments
- (88) Date of publication of the international search report: 8 June 2006

For two-letter codes and other abbreviations, refer to the "Guid-(74) Agent: G.E. EHRLICH (1995) LTD.; 11 Menachem Be- ance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: METHODS OF GENERATING STEM CELLS AND EMBRYONIC BODIES CARRYING DISEASE-CAUSING MU-TATIONS AND METHODS OF USING SAME FOR STUDYING GENETIC DISORDERS

(57) Abstract: Stem cells, stem cell lines and differentiated cells, tissues and organs which carry disease-causing mutations are provided. There is also provided a method of identifying agents suitable for treating disorders associated with at least one diseasecausing mutations such as myotonic dystrophy and van Waardenburg syndrome.